OUTCOMES-BASED ARRANGEMENTS

Sustainable Financing for Transformative Therapies and a Review of State Activity

APRIL 2023

A CAMPAIGN FOR Transformative Therapies

Executive Summary

As newly developed, innovative therapies come to market, policymakers continue to explore sustainable financing solutions to pay for these life-changing medicines. Outcomes-based arrangements (OBAs) - which base reimbursement on whether or not a therapy worked- are one tool to help manage costs. This is particularly applicable to gene therapies for rare diseases, where the pipeline is robust, and the need is great. The U.S. federal government has taken steps through rulemaking to enable these arrangements. Some states are leading the way through Medicaid State Plan Amendments (SPAs), with Oklahoma Medicaid implementing some of the earliest OBAs. As more states investigate financing options for gene therapies, this paper reviews example OBAs and key lessons learned from states with existing contracts.

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Introduction

The United States spends more on healthcare than any other country.[1] Yet despite this spending, life expectancy in the United States trails behind other developed countries.[2] To alter this trajectory, we must re-frame how care is reimbursed and build a more sustainable financing system that pays for value.

A major contributor to the current state of the U.S. healthcare system is the widespread use of traditional managed care models that restrict access to costly treatments through utilization management and other techniques. These models use cost as the key metric to control access. As systems and technology have evolved, alternative systems that encourage innovation by paying for value rather than restricting access based on cost alone, are emerging.

One such method is the implementation of outcomes-based arrangements (OBAs), where payments are tied to the results - or value - of a therapy or procedure rather than volume. These OBAs can be applied to various healthcare models and prescription drugs - in particular, gene therapies are ripe for changes in reimbursement. This is due to multiple factors: the rare nature or unmet need of some of the conditions treated by these gene therapies, the relatively high cost of such treatments, and the need to collect and analyze clinical data regarding outcomes and durability.

ALSO KNOWN AS (AKA)

VALUE-BASED ARRANGEMENT (VBA): Catch-all term for any type of innovative contracting where price is based on some definition of value or quality; often used for provider payments

OUTCOMES-BASED ARRANGEMENT (OBA): Generally used for prescription drugs, OBAs base payment on patient outcomes

VALUE-BASED PURCHASING ARRANGEMENT (VBP): Official U.S. government (CMS) definition targeted to prescription drugs; agreement that aligns payment to an observed or expected therapeutic or clinical value (outcomes relative to costs) in a population

Gene therapies are already transforming care delivery and improving patient lives in ways previously thought to be beyond our capabilities. By addressing non-functioning genes (often in a single prescribed course of treatment), gene therapies target the underlying cause of disease and can potentially make long-lasting changes that transform patients'

1 In 2019, the United States spent nearly 18 percent of its Gross Domestic Product (GDP) on national health expenditures, totaling $3.8 trillion dollars; available: https://www.cms.gov/Research-Statistics-Data-and-Systems/Statistics-Trends-and-Reports/NationalHealthExpendData/NHE-Fact-Sheet.

2 In 2019, life expectancy at birth in the U.S. was 78.9 years, which is the lowest among countries with high GDP per capita; from Kaiser Family Foundation's Health System Tracker; available: https://www.healthsystemtracker.org/chart-collection/u-s-life-expectancy-compare-countries/

lives. Patients may be able to engage in activities they never thought possible, often achieving basic functions that most of us take for granted in our everyday lives.

Given the significant value these therapies can offer patients, caregivers, and the healthcare system, coupled with the realities of complex research and development programs and relatively small patient populations,[3] the list price can also be significant. With seven gene therapies currently[4] on the market in the U.S. and more in development[5], these transformative therapies may quickly become commonplace for certain disease states. To deliver on the promise to help patients while recognizing the financial constraints of many payers, including state Medicaid programs, the time to develop public policy to apply sustainable financing models - like OBAs - is now. Conversely, to ignore the pipeline of costly life-changing treatments that are on the horizon will result in fragmented policies that will likely restrict patient access and fail to build the concept of value into financing mechanisms.

How a particular state Medicaid program moves forward to implement OBAs can take different paths, which are outlined in greater detail below. These steps include the process laid out in the Centers for Medicare & Medicaid Services (CMS) Medicaid VBP Rule; submission of a state plan amendment (SPA) requesting authority to enter into OBAs; and/or passage of state legislation, where required.

The goal of this paper is to provide greater understanding of the current landscape of OBAs among public payers, focusing on lessons learned from existing agreements within state Medicaid programs and exploring the regulatory environment for such arrangements.

Policy Environment

Value-based reimbursement achieves cost savings from improved disease management that targets results (e.g., did the patient improve?) rather than procedures (e.g., did the patient receive the service as prescribed?). Conceptually, this means that payers would only reimburse for treatments that work in the manner expected. Even in today's polarized political environment, many policymakers and thought leaders have expressed the need to better incentivize effective care for patients. To that end, several strategies have emerged to transition the current system toward value-based care - such as the Center for Medicare and Medicaid Innovation (the Innovation Center) Enhanced Oncology Care Model[6], Bundled Payments for Care Improvement Initiative[7], and Next Generation Accountable Care Organizations[8]. In addition, Medicaid programs are increasingly

3 Recent projections indicate an average of 93,000 patients will be treated by cell and gene therapies by 2030, which is a fraction of one percent of the population; Young, CM, et al. Durable cell and gene therapy potential patient and financial impact: U.S. projections of product approvals, patients treated, and product revenues; Drug Discovery Today; 27(1)17-30; 2020; available: https://www.sciencedirect.com/science/article/pii/S1359644621003901?via%3Dihub

4 As of Q1 2023, the following gene therapies have been approved in the U.S.: ADSTILADRIN®, HEMGENIX®, IMLYGIC®, LUXTURNA®, SKYSONA®, ZOLGENSMA®; and ZYNTEGLO®; available: https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products

5 There are currently over 2,000 gene therapies in clinical development; available from the American Society of Gene and Cell Therapies Q2 2022 Quarterly Data Report; available: https://asgct.org/global/documents/asgct-pharma-intelligence-quarterly-report-draft-q.aspx

6 https://innovation.cms.gov/innovation-models/enhancing-oncology-model

7 https://innovation.cms.gov/innovation-models/bundled-payments

8 https://innovation.cms.gov/innovation-models/next-generation-aco-model

implementing value-based strategies with the Centers for Medicare & Medicaid Services (CMS) offering technical assistance to states for value-based payment approaches through their Innovation Accelerator Program.[9]

Until recently[10], prescription drugs have been left out of this transition to value-based care as government-initiated reforms have focused on services, with drugs being an afterthought or specifically carved out.[11] This has left pharmaceutical manufacturers (manufacturers) and payers (both public - i.e., state Medicaid programs - and private - i.e., commercial plans) on their own to develop OBA models.[12]

These models have had some success in the private sector with calls for expansion into federal programs (i.e., Medicare and Medicaid). However, barriers still exist and the resulting advocacy around prescription drug OBAs encouraged the federal government- under two different Administrations- into acting. CMS, under the Trump Administration, promulgated a Medicaid VBP Rule at the end of 2020,[13] which went into effect in July 2022. That rule is intended to facilitate value-based payments for prescription drugs (discussed in more detail on page 9). Then in October 2022, President Biden released an Executive Order[14] that directed CMMI to develop and test models for reducing prescription drug costs that also improve access, specifically referencing value-based payments. As a result of that Executive Order, in February 2023, HHS announced that CMMI would test a Cell and Gene Therapy Access Model[15] that would coordinate and administer multi-state OBAs for certain cell and gene therapies as voluntarily requested by state Medicaid agencies.

While the federal government is implementing its various policies, state Medicaid programs have taken charge. Medicaid spending on molecular targeted therapies doubled between 2015 and 2019 and accounted for the fifth most costly drug group in 2019.[16] This trend is expected to continue if not addressed due to the robust pipeline of transformative therapies currently in development. To this end, fourteen state Medicaid programs have already received approval from CMS for State Plan Amendments (SPAs)[17] that enable them to enter into OBAs with manufacturers, and additional states have recently requested clearance to participate.

9 https://www.medicaid.gov/resources-for-states/innovation-accelerator-program/functional-areas/value-based-payment/value-based-payment-financial-simulations/index.html

10 February 2023 CMMI Announcement here: https://innovation.cms.gov/data-and-reports/2023/eo-rx-drug-cost-response-report ; and July 2022 implementation of 2020 CMS VBP Rule here: https://www.medicaid.gov/prescription-drugs/downloads/mfr-rel-116-vbp.pdf

11 The Office of the Inspector General (OIG) and Centers for Medicare and Medicaid Services (CMS) finalized regulations that created new safe harbors under the Anti-Kickback Statute (AKS) and Stark Laws for certain value-based arrangements but specifically carved out pharmaceutical manufacturers and pharmacy benefit managers from participating in these arrangements, thereby omitting prescription drug OBAs; OIG rule accessed here: https://www.federalregister.gov/documents/2020/12/02/2020-26072/medicare-and-state-health-care-programs-fraud-and-abuse-revisions-to-safe-harbors-under-the; CMS rule accessed here: https://www.federalregister.gov/documents/2020/12/02/2020-26140/medicare-program-modernizing-and-clarifying-the-physician-self-referral-regulations#sectno-reference-411.351

12 https://phrma.org/-/media/Project/PhRMA/PhRMA-Org/PhRMA-Org/PDF/P-R/PhRMA_InnovativeContracts_Sep2020.pdf

13 https://www.federalregister.gov/documents/2020/12/31/2020-28567/medicaid-program-establishing-minimum-standards-in-medicaid-state-drug-utilization-review-dur-and

14 https://www.whitehouse.gov/briefing-room/presidential-actions/2022/10/14/executive-order-on-lowering-prescription-drug-costs-for-americans/

15 https://innovation.cms.gov/data-and-reports/2023/eo-rx-drug-cost-response-report

16 https://www.kff.org/medicaid/issue-brief/utilization-and-spending-trends-in-medicaid-outpatient-prescription-drugs-2015-2019/

17 https://www.medicaid.gov/medicaid/medicaid-state-plan-amendments/index.html

Outcomes-Based Arrangements 101

What is an OBA?

Outcomes-based arrangements (OBAs) are agreements between payers and manufacturers that tie reimbursement to a drug's effectiveness. Should it be determined the drug is not successful because the patient did not meet predetermined outcomes, the manufacturer rebates, refunds, or repays money back to the payer. Payers would only be responsible for paying for therapies that improve patient outcomes, which would reduce wasteful spending in the system by delivering the right care to the right patient at the right time.

How do OBAs Work?

Feasibility and implementation are centered on a contractual arrangement between the manufacturer and the payer[18] where a patient's outcome determines the ultimate reimbursement rate. According to the contract, should the drug prove successful for the patient, the manufacturer retains the original payment. Conversely, should the drug fail to achieve the agreed-upon outcomes, the manufacturer would provide additional rebates, refunds, or repayments to the payer (potentially amounting to a significant portion of the Wholesale Acquisition Cost or WAC), depending on the terms of the contract.

This model stands in direct contrast to the traditional approach for pharmaceutical reimbursement, which incentivizes prescription volume rather than patient outcomes; manufacturers and prescribers are reimbursed per pill regardless of the results. Such practices lack focus on coordination of care and, therefore risk truncated and inefficient patient care, suboptimal clinical results, and large bills for payers and patients. Conversely, OBAs incentivize delivery of the right treatment to the right patient population (where the treatment is most likely to be effective), thus minimizing rebates, refunds, or repayments from the manufacturer.

This is especially noteworthy due to the recent trend toward the development and prescribing of more targeted drug therapies for smaller patient populations, which tend to be more expensive than traditional therapies for larger populations. Under the current system, even a potentially high value drug can be labeled "low value" due to its high price tag and limited number of impacted patients (often those with rare medical conditions), and consequently can lead insurers to implement measures that restrict access to those products. Since OBAs incentivize the right drug for the right patient, there is a higher likelihood of lower downstream costs by minimizing exacerbated medical conditions, which mitigates the perceived need for access restrictions payers may implement.

18 OBAs can also be contractual agreements between providers and manufacturers or between providers and payers.

How are Outcomes Tracked?

To ensure value for patients, clearly defined and measurable metrics can be used to judge the performance of a particular drug or treatment. As referenced, OBAs can condition payment on a variety of outcomes such as medication adherence, reduced rates of hospitalization, or certain biomarkers such as reduction of tumor size for specific cancers or the amount of clotting factor used for some bleeding disorders.

Claims data are the most common method for tracking outcomes today. However, using only claims data limits the type of outcome information that can be collected. De-identified patient-level clinical data captured in electronic health records (EHR) or submitted by a provider to a third party can also be used, but the barriers around data sharing, administrative burden, and privacy concerns make this more difficult. However, as technology and regulations around interoperability and data sharing evolve, the promise for OBAs to yield even greater savings based on more specific patient outcomes is significant.

Regulatory Barriers to Outcomes-Based Arrangements

As transformative therapies continue to receive approval from the Food & Drug Administration (FDA) and as states further explore alternative payment mechanisms, both public and private payers are increasingly interested in OBAs and similar models that incorporate value in payments to drug manufacturers. However, one of the largest and most often cited deterrents to the widespread use of OBAs is the best price[19] provision of the Medicaid Drug Rebate Program (MDRP). This provision stipulates that Medicaid should pay no more than the lowest price a manufacturer offers to any other provider, payer, or retailer (with some caveats). If a manufacturer enters into an OBA and provides a rebate, refund, or repayment to a private payer for just one non-responding patient, that would lower the best price of the drug and require the manufacturer to provide the drug at the new low price to the entire Medicaid population, regardless of patient outcomes. This discourages manufacturers from offering large rebates, refunds, or repayment terms as part of their OBAs.

Similarly, both the Average Manufacturer Price (AMP)[20] and Average Sales Price (ASP)[21] must also be addressed regarding how they are calculated for drugs that have an OBA. The AMP is the average price paid to a manufacturer by wholesalers for drugs distributed to retail pharmacies. The ASP is calculated based off the sales from manufacturers to all purchasers, including discounts. The federal government uses AMP to determine how manufacturers rebate drugs in Medicaid[22] and ASP for Medicare Part B (physician-administered) drugs, Unless OBAs are exempted from both price reporting calculations (AMP and ASP), the potential rebates, refunds, or repayments due for non-responding patients could artificially skew how AMP and ASP are reported.

19 42 U.S.C. § 1396r-8(c)(1)(C)
20 42 U.S.C. § 1396r-8(k)(1)(A)
21 42 U.S.C. § 1395w-3(a)
22 AMP also serves as the basis for 340B pricing.

Lastly, the fraud and abuse laws including Stark[23] and the Anti-Kickback Statute (AKS)[24] may present barriers for OBAs. Stark prohibits a physician from making referrals for certain designated health services, which include prescription drugs, that are reimbursed by CMS. The AKS prohibits providing anything of value to induce the purchase of items or services reimbursed by federal healthcare programs. Since both laws are fairly broad in their application, there is some concern OBAs could trigger them regarding the refunds, rebates, or repayments provided (i.e., the Office of the Inspector General [OIG] could view the refunds as "something of value" and all parties participating in the OBA could be subject to criminal prosecution unless OBAs are clearly defined in a safe harbor).

CMS Efforts to Promote Adoption of Value-Based Payments for Drugs

CMS Medicaid VBP Rule

In response to the regulatory and statutory obstacles that undermined the agency's support for more widespread adoption of value-based payments, CMS promulgated regulations[25] at the end of 2020, which went into effect in July 2022, to provide flexibility to manufacturers and payers, including state Medicaid programs, to enter into OBAs without triggering the best price provision (note: CMS defines OBAs as value-based purchasing agreements or VBPs). There are ongoing federal efforts to seek additional clarification, either through additional guidance, rulemaking, or legislation, about the CMS VBP rule to support this avenue for implementing OBAs.

The CMS rule enables manufacturers to report multiple best prices for prescription drugs that have a VBP in place: one or more[26] best price(s) for patients when the drug successfully achieved the desired outcome(s) (called "responders"); another best price for cases where the drug did not work as intended ("non-responders"); and another best price for drugs not part of a VBP. These multiple best prices would be made available to states that are willing to adhere to the same VBP arrangement terms as are in place in the commercial sector, which would provide those states with the option to receive the benefits of the arrangements already available in the marketplace. States are not required to go through the SPA process or obtain CMS approval to enter into VBP arrangements with manufacturers under this multiple best price approach.

It is estimated that the uptake of the new VBP arrangements will save states and the federal government $228 million over three years.[27] But these arrangements deliver value beyond taxpayer savings, including reduced overall patient medical spending, reduction in disease progression and/or improvement of symptoms, or improvement in reported quality of life. Most importantly, for patients living with serious medical conditions, access to new therapies can be lifesaving and life changing.

23 42 U.S.C. § 1395nn

24 42 U.S.C. § 1320a-7b

25 Medicaid Program: Establishing Minimum Standards in Medicaid State Drug Utilization Review and Supporting Value-Based Purchasing for Drugs Covered in Medicaid, Revising Medicaid Drug Rebate and Third Party Liability Requirements, 85 Fed. Reg. 87000 (December 31, 2020).

26 Hypothetically, one OBA could have a tiered rebate system with multiple best prices depending on a range of effectiveness observed in the patient post-treatment.

27 https://www.cms.gov/newsroom/press-releases/cms-issues-final-rule-empower-states-manufacturers-and-private-payers-create-new-payment-methods

MULTIPLE BEST PRICE EXAMPLE

Drug X treats cancer, and the OBA is based on tumor size. If X does not shrink a patient's tumor by 100% in 1 year, manufacturer rebates payer 100%.

- **BEST PRICE #1:** $1000 for patients with 100% tumor reduction
- **BEST PRICE #2:** $0 for patients with <100% tumor reduction
- **BEST PRICE #3:** $800* for non-VBP patients

*The non-VBP price is hypothetically calculated based on the existing formula that takes into account the price concessions available anywhere in the marketplace.

Regarding AMP, the VBP rule does not directly address how it is calculated when a prescription drug has a VBP in place. In the final rule, CMS specifically discusses AMP with respect to payments made over time[28] as they relate to VBPs but not for VBP upfront payments. Since many rare disease gene therapies will be sold with federally-mandated rebates or discounts, accuracy in calculating these payments is paramount. This lack of clarity around AMP and implementation timelines, combined with the uncertainty around how different administrations may interpret these regulations, has resulted in a federal advocacy effort where interested parties are advocating for legislation and regulations that provide additional clarity.

CMMI CGT Demonstration

In October 2022, President Biden issued an Executive Order[29] directing CMMI to develop and submit a report describing potential models for lowering drug costs and promoting access to innovative drug therapies for Medicare and Medicaid beneficiaries. HHS then released a report in February 2023 selecting three new payment models for testing through CMMI, one of which is the Cell and Gene Therapy Access Model (CGT Model).[30]

The Medicaid-focused CGT Model would establish a partnership among CMS, manufacturers, and state Medicaid agencies that tests a new, centralized approach to facilitate OBAs for certain cell and gene therapies where CMS would act as the administrator of the program.

As of March 2023, additional details around the CGT Model are missing other than a goal implementation date of 2026, which is receiving push back from policymakers, industry, and patient groups for being too slow. This demonstration is also an indication that CMS and the Biden Administration are interested in pursuing policies that promote OBAs, but there is still work to be done regarding the ongoing federal barriers these new payment models face.

28 https://www.federalregister.gov/d/2020-28567/p-246

29 https://www.whitehouse.gov/briefing-room/presidential-actions/2022/10/14/executive-order-on-lowering-prescription-drug-costs-for-americans/

30 https://innovation.cms.gov/data-and-reports/2023/eo-rx-drug-cost-response-report

State Actions to Leverage OBAs in Medicaid

While covering prescription drugs is optional under Medicaid, all 50 states have opted to provide this benefit.[31] As part of the benefit, states must cover all drugs from manufacturers participating in the MDRP. Therefore, states may face increasing financial pressure as they seek to cover new, potentially high-cost, and life-altering therapies. This challenge will only become more acute as the availability of new therapies grows over the next five to ten years. Given the atmosphere of rising prescription drug costs and the pipeline of new transformative therapies, states are seeking new ways to mitigate the budgetary impact.

Within this broader policy context, each state has unique approaches to its Medicaid system. Some states have relied on managed care organizations (MCOs) to make decisions on the medical effectiveness of a particular drug for a particular patient, leading to potentially inconsistent treatment for clinically similar patients. In other cases, the managed care decisions are made inside the state Medicaid department, which adds consistency but increases complexity. This means the system can be particularly complicated for high-risk patients with high-cost medical conditions because their treatment options may vary significantly depending on the structure of the Medicaid program and the managed care criteria used to evaluate treatment options. Layered on top of this are the different ways Medicaid programs purchase prescription drugs and specialty therapies. Such methods add further complexity as each state evaluates access to such therapies.

TYPES OF STATE Rx VALUE-BASED ARRANGEMENTS

SUBSCRIPTION-BASED MODELS: State pays a flat fee to a drug manufacturer for unlimited access to a specific drug. In turn, the state must agree to let that manufacturer be the sole provider of said drug.

OUTCOMES-BASED AGREEMENT MODELS: Manufacturers provide the state supplemental rebates, if agreed upon clinical outcomes are not met.

WARRANTY AGREEMENTS*: Some manufacturers are developing alternative models like warranty agreements, whereby third parties other than the manufacturer may make payments to states when patients do not meet predetermined outcomes.

*No state warranty agreements are in place as of Q1 2023.

31 https://www.ncsl.org/research/health/medicaid-pharmaceutical-laws-and-policies.aspx

Since limiting the scope of covered drugs to control costs is prohibited under the federal statutory rebate agreement outlined in the MDRP, and undesirable from a patient access perspective, states are increasingly considering value-based approaches to both manage costs and improve patient health outcomes.

STATE MEDICAID OPTIONS FOR ESTABLISHING Rx VBAs

CMS VBP RULE*: States would review available VBP arrangements on the CMS Medicaid Drug Programs (MDP) system and work with individual manufacturers on implementation. This does not require a state plan amendment.

STATE PLAN AMENDMENT: States would submit a SPA to CMS requesting authority to adopt alternative payment methods for prescription drugs. State Medicaid programs would then individually reach out and negotiate with interested manufacturers over specific prescription drugs.

STATE LEGISLATION: Some states require legislation to be passed prior to pursuing a SPA. See Appendix A for examples.

CMMI DEMONSTRATION: TBD. CMMI is developing a pathway for states to work with CMS and manufacturers to establish and implement VBPs but details have not been released on next steps.

*https://www.medicaid.gov/prescription-drugs/downloads/state-rel-189-vbp.pdf

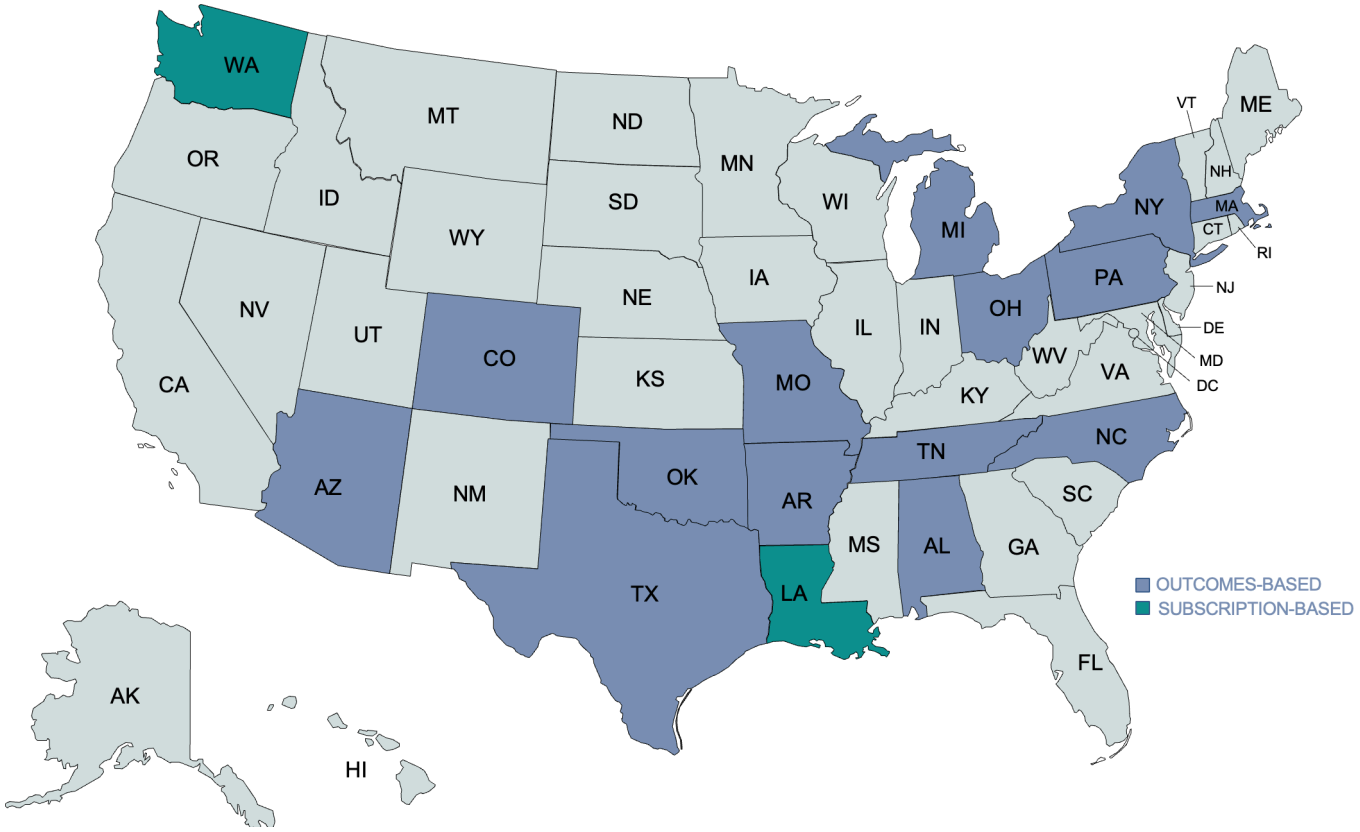
For example, ZOLGENSMA® is a gene therapy that targets the genetic root cause of spinal muscular atrophy (SMA), a devastating disease that results in severe and often deadly muscle weakness that makes breathing, eating, and moving extremely difficult. At early-onset, SMA is the most common genetic cause of infant mortality.[32] ZOLGENSMA is a one-time infusion into a vein targeted to treat children less than two years old with severe SMA by replacing the faulty gene with a new working copy, stopping progression of the disease.[33] Most state Medicaid programs cover ZOLGENSMA; however, with a price tag of $2.1 million[34] per dose, states generally require several steps before authorizing use. Therefore, some states are turning to OBAs as a method to ensure the therapy is worth the investment. As noted by then-Massachusetts Medicaid Director Dan Tsai, "We think it makes sense to pay for innovation for a drug that could really work. And we think [Massachusetts], with public dollars, should not pay for something if it doesn't do what it's advertised to do."[35]

As previously discussed, some state Medicaid agencies have already submitted proposals to the federal government in the form of State Plan Amendments (SPAs) to CMS to adopt alternative payment methods through OBAs. Generally speaking, if the OBA state proposal is approved by CMS, "the state and each manufacturer are able to jointly agree on benchmarks based on health outcomes and the specific populations for which these outcomes-based benchmarks will be measured and evaluated."[36]

32 https://www.zolgensma.com/how-zolgensma-works
33 Ibid.
34 https://www.novartis.com/news/media-releases/avexis-announces-innovative-zolgensma-gene-therapy-access-programs-us-payers-and-families
35 https://www.wbur.org/news/2020/02/07/massachusetts-masshealth-zolgensma-cost-control
36 https://www.cms.gov/newsroom/press-releases/cms-approves-state-proposal-advance-specific-medicaid-value-based-arrangements-drug-makers

As of March 2023, sixteen states have received approvals for their SPAs to enable negotiation of VBP contracts with drug manufacturers, while a few additional states await approval.

STATES WITH APPROVED SPAs



Thus far, for the approved SPAs, two primary payment models have been proposed: (1) subscription-based purchasing, and (2) outcomes-based supplemental rebate agreements. Twelve states (AL, AZ, AR, CO, MA, MI, MO, NY, NC, OH, OK, PA, TN, TX) reported adding outcomes-based supplemental rebate language to their Medicaid statutes, while the remaining two states (LA, WA) proposed modified subscription models for hepatitis C antiviral drugs.

Although states received approval from CMS to begin negotiations of VBPs in their drug purchasing programs, only a few states have publicly announced such contracts with drug manufacturers. Examples of state OBAs are detailed below:

COLORADO: Colorado utilizes supplemental rebates to design and implement outcomes-based agreements.

- **AveXis (Novartis)[37]:** Contracted with AveXis (Novartis Gene Therapies) for its gene therapy drug ZOLGENSMA® (onasemnogene abeparvovec-xioi), used to treat spinal muscular atrophy (SMA) in children under 2 years old. This is a one-time treatment with a list price of $2.1 million per treatment. Under this model, the Colorado Department of Health Care will be able to receive back a significant portion of the price if the therapy is not successful in delivering the expected clinical health outcomes for a five-year period following its use.

OKLAHOMA: Oklahoma currently leads as the state with the most existing OBA contracts with drug manufacturers.

- **Melinta[38]:** Contracted with Melinta Therapeutics for the antibiotic ORBACTIV® (oritavancin), an antibacterial treatment for skin infections. Historically, ORBACTIV is more expensive than other treatments and the state used prior authorization as a cost management tool, slowing access to the drug. However, despite the higher cost, if used as a first-line treatment, ORBACTIV promises lower overall costs by avoiding hospitalizations. Under the terms of the OBA, the state will no longer use prior authorization for ORBACTIV and Melinta is responsible (via higher rebates) if patients incur higher costs due to hospitalizations.
- **Alkermes[39]:** Contracted with Alkermes for ARISTADA® (aripiprazole lauroxil), an injectable treatment for schizophrenia. Oklahoma's OBA for ARISTADA aims to improve patient adherence to the treatment plan by decreasing the monthly prescription cost.

LOUISIANA: Louisiana's arrangements with pharmaceutical manufacturers are volume-based rather than value-based, utilizing a subscription-based model.

- **Asegua (Gilead)[40]:** In 2019, contracted with Asegua Therapeutics, a subsidiary of Gilead Sciences Inc., for a hepatitis C treatment, the authorized generic of EPCLUSA® (sofosbuvir/velpatasvir), via a subscription-based model. Under this model, the state and manufacturer agree to an aggregate cap on the costs of this medication regardless of volume. This provides the state with predictable budgetary impacts while ensuring patient access.

37 Williams, M. (2022) Colorado Medicaid Executes Its First Pharmaceutical Value-Based Contracts. Colorado Department of Health Care Policy & Financing. https://hcpf.colorado.gov/colorado-medicaid-executes-its-first-pharmaceutical-value-based-contracts

38 Beck, J. (2018). Oklahoma Signs the Nation's First State Medicaid Value-Based Contracts for Rx Drugs. National Academy for State Health Policy. https://www.nashp.org/oklahoma-signs-first-medicaid-value-based-contracts-for-rx-drugs/

39 https://www.reuters.com/article/us-health-usa-medicaid/oklahoma-medicaid-tests-new-tactic-to-curb-u-s-drug-costs-idUSKCN1L81L6

40 Louisiana Department of Health (2019). State's innovative payment agreement with Asegua Therapeutics for hepatitis C medication allows more Louisianans to receive life-saving treatment. https://ldh.la.gov/index.cfm/newsroom/detail/5357

WASHINGTON: Similar to the Louisiana model, Washington also employs a subscription-based model.

- **AbbVie[41]:** Engaged with AbbVie in a public-private partnership that relies on a modified subscription model to help the state control costs related to hepatitis C. Similar to Louisiana, the state receives unlimited access to AbbVie's hepatitis C drug, MAVYRET® (glecaprevir/pibrentasvir), for a capped cost. Unique to Washington, however, the public-private partnership takes the subscription model further by coordinating broader public health efforts around eliminating hepatitis C among Washington's Medicaid program (Washington State Health Care Authority), Washington State Department of Health, AbbVie, and community leaders.[42]

ARIZONA: Arizona utilizes supplemental rebates to design and implement outcomes-based agreements.

- **AveXis (Novartis)** [43]: Engaged with AveXis, a Novartis company, to implement a value-based contract for ZOLGENSMA, an innovative gene therapy for pediatric patients with spinal muscular atrophy. The model is based on rebates paid back to the state if certain agreed-upon patient outcomes are not met.

Please see Appendix B for additional details, including individual state profiles for states that have a SPA approved by CMS to engage in OBAs with pharmaceutical manufacturers.

Lessons Learned from Existing State OBAs

In researching state initiatives around managing prescription drug costs through OBAs, the Campaign for Transformative Therapies (CTT) interviewed a subset of states regarding their OBA contracts, lessons learned, and wish lists for future potential contracts. Below, recurring themes and observations are listed to better inform future contracts between payers (e.g., additional states) and manufacturers.

1. **Data collection and reporting are challenging.**
   The most common avenue for analyzing the results of OBAs is through claims data. Clinical data at the individual patient level are difficult to collect, and there is no common method for collecting these data among states - some states are using third parties for the tracking and reporting of data; some states are allowing the pharmaceutical manufacturers to handle the data collection; and some states are performing these functions on their own. Claims data are the easiest data to analyze because they are widely available, objective, and standardized; however, most states agree that using claims data often fails to accurately reflect patient outcomes and there may be a significant time lag in the claims submission process that limits analysis. The more robust clinical data collected in electronic health records by providers is clearly preferable; however, this requires a practical mechanism to collect and share the data. Interestingly, there was no consensus regarding control over the data: some states prefer internally maintaining such control while others see value in the manufacturers

41 https://stories.abbvie.com/stories/for-hepatitis-c-seeking-end-road-in-washington-state.htm
42 https://www.spokesman.com/stories/2021/mar/05/michael-ninburg-and-sue-birch-a-public-private-par/
43 Arizona Bioindustry Association (2020). Curing the Incurable – This Gene Therapy Delivers. https://www.azbio.org/curing-the-incurable-this-gene-therapy-delivers

retaining this function. This could be due to operational components of each state's Medicaid program, as well as the varying relationships between Medicaid programs and manufacturers. A third option also emerged, where the manufacturers pay for a third party to collect the data. However, in the event manufacturers are compelled to pay for the collection of data, they must consider compliance with the AKS in addition to privacy laws.

2. **States want more meaningful outcomes to be part of OBAs.** Some states want to see measured outcomes that reflect functional outcomes. For example, for ZOLGENSMA, rather than measuring survival (i.e., did the child live past two, three, four years?), some states expressed the desire to know if a child was able to sit, walk, or eat by themselves when they were previously unable to achieve such milestones. Another example is LUXTURNA® (voretigene neparvovec-rzyl), which treats vision loss. Commercial market OBAs for LUXTURNA are often based on light sensitivity tests.[44] However, states have suggested that knowing/reporting whether the patient is able to navigate better in daily life would be an improved incentive for states to invest time and effort into developing OBAs for this therapy.[45] On the other hand, manufacturers may be reluctant to enter into arrangements where the reported outcomes were not studied extensively in clinical trials or in subsequent real-world evidence studies post-FDA approval. This could hypothetically be addressed through supplemental contractual language or reports that include mutually agreed upon metrics regarding patient outcomes.

3. **States are interested in pursuing additional OBAs, with caveats.** Generally, states expressed interest in pursuing OBAs with additional manufacturers. At the same time, concerns over the significant time and effort required by state Medicaid programs to invest and build an arm of their department aimed at negotiating with manufacturers over OBAs, as well as the additional work and functional expertise necessary to maintain and track ongoing OBAs were recurring themes. Some states with approved SPAs have not yet executed an OBA because of these issues, yet they maintain a willingness to continue engaging with manufacturers on finalizing future contracts. Other states that may have only one OBA in place and are satisfied with the results thus far are finding it difficult to increase the utilization due to a lack of manufacturers willing to engage, in part because of existing legal and regulatory concerns. Still, other states that do not have OBAs in place are interested in the process but want manufacturers to provide them with unique terms that fit their populations rather than a "one-size-fits-all-states" approach. A minority of states claim that they would be interested in pursuing additional OBAs if manufacturers' risks were greater.

Despite the hurdles for implementing OBAs, the general trend indicates that more states are interested in pursuing OBAs as one method for controlling high drug costs. Since each state Medicaid program is unique in how they design the drug benefit (e.g., managed care vs. fee-for-service, various models for dealing with high-cost specialty drugs, etc.) and have different budget cycles (e.g., two years vs. one year), each state OBA is unique and must be approached as such. This theme produced another suggestion: creation of a public database maintained by CMS that lists the various OBAs states have in place. The information would need to exclude proprietary details. This could provide states with a menu of potential approaches to utilize depending on their patient populations and programmatic structures. CMMI might consider this approach as it develops its CGT Model.

44 https://www.harvardpilgrim.org/public/news-detail?nt=HPH_News_C&nid=1471914707173

45 2021 CTT interviews with state Medicaid programs

Appendix A: Example State Legislation

Some states may find it necessary or preferable to adopt legislation which would permit implementation of OBAs prior to submitting a State Plan Amendment (SPA) to CMS. Such legislation has been adopted in Texas and Ohio. Below is model bill language "relating to value-based arrangements in the Medicaid vendor drug program."

Texas S.B.1780 (86th Legislature); Passed on 5/28/2019[46]

AN ACT
relating to value-based arrangements in the Medicaid vendor drug program.
BE IT ENACTED BY THE LEGISLATURE OF THE STATE OF TEXAS:

SECTION 1. Subchapter B, Chapter 531, Government Code, is amended by adding Section 531.0701 to read as follows:

Sec. 531.0701. VALUE-BASED ARRANGEMENTS. (a) In this section, "manufacturer" has the meaning assigned by Section 531.070.

(b) Subject to Section 531.071, the commission may enter into a value-based arrangement for the Medicaid vendor drug program by written agreement with a manufacturer based on outcome data or other metrics to which this state and the manufacturer agree in writing. The value-based arrangement may include a rebate, a discount, a price reduction, a contribution, risk sharing, a reimbursement, payment deferral or installment payments, a guarantee, patient care, shared savings payments, withholds, a bonus, or any other thing of value.

SECTION 2. If before implementing any provision of this Act a state agency determines that a waiver or authorization from a federal agency is necessary for implementation of that provision, the agency affected by the provision shall request the waiver or authorization and may delay implementing that provision until the waiver or authorization is granted.

SECTION 3. The Health and Human Services Commission is required to implement a provision of this Act only if the legislature appropriates money specifically for that purpose. If the legislature does not appropriate money specifically for that purpose, the commission may, but is not required to, implement a provision of this Act using other appropriations available for that purpose.

SECTION 4. This Act takes effect September 1, 2019.

46 https://legiscan.com/TX/text/SB1780/2019

Ohio H.B. 110 (134th General Assembly); Passed on 7/1/202147

SECTION 333.215. VALUE-BASED PURCHASING SUPPLEMENTAL REBATE

(A) Not later than sixty days after the effective date of this section, the

Department of Medicaid shall submit to the United States Centers for Medicare and Medicaid Services a Medicaid state plan amendment to authorize the Department to enter into value-based purchasing supplemental rebate agreements with pharmaceutical manufacturers.

(B) The agreements authorized by the state plan amendment shall establish criteria for the payment of supplemental rebates. The Department of Medicaid shall use its best efforts to ensure that the form value-based supplemental rebate agreement submitted to the Centers for Medicare and Medicaid Services permits rebates to be calculated on many different bases at the discretion of the Department with the approval of the pharmaceutical manufacturer, including under outcome-based models, shared savings Am. Sub. H. B. No.110 134th G.A. 2233 models, subscription or modified subscription models, risk-sharing models, or guarantees. The rebates may be calculated and paid in a single year or over multiple years.

(C) Nothing in this section requires a drug manufacturer or the Department to enter into a supplemental rebate agreement under this section.

47 https://ohiohouse.gov/legislation/134/hb110

Appendix B: Profiles for States with Approved OBA-Type State Plan Amendments

GLOSSARY OF TERMS

ACA		Affordable Care Act
DUR		Drug Utilization Review Board
FFS/MCO		State hybrid Medicaid model that utilizes both traditional fee-for-service (FFS) and managed care organizations (MCOs)
FFS		Fee-for-service
MCO		Managed care organization
P&T Committee		Pharmacy & Therapeutics Committee
PBM		Pharmacy Benefit Manager
Pharmacy Benefit	**Carve in**	Some states carve the benefit into managed care
	Carve out	A few states completely carve out the benefit from managed care and handle all drug pricing within the state Medicaid program
	Hybrid $$$ Rx	Some states take a hybrid approach and only carve out the most expensive or specialty drugs but leave the majority of drugs carved into managed care
PDL		Preferred drug list; a list of outpatient drugs that states encourage prescribers to prescribe over others
UPDL		Uniform preferred drug list; states that use MCOs to administer pharmacy benefits may use a uniform preferred drug list that requires all MCOs to cover the same drugs as FFS
Rx		Prescription drug

DISEASE POPULATION ESTIMATES BASED ON:

- **CYSTIC FIBROSIS:** Cystic Fibrosis Foundation; 2021 Cystic Fibrosis Foundation Patient Registry Highlights Report; https://www.cff.org/media/26631/download
- **DIABETES:** American Diabetes Association State Fact Sheets; https://diabetes.org/about-us/statistics/by-state
- **HEMOPHILIA:** CDC; https://communitycountsdataviz.cdc.gov/blooddisorders/#!/
- **ACUTE HEPATITIS C:** CDC 2019 data based on reported cases; https://www.cdc.gov/hepatitis/statistics/2020surveillance/hepatitis-c/figure-3.3.htm
- **SICKLE CELL DISEASE:** Sick Cells State Map; https://sickcells.org/advocacy-tools/
- **SPINAL MUSCULAR ATROPHY (SMA):** Cure SMA State Fact Sheets; https://www.curesma.org/advocacy/#state-fact-sheets

SPA VBP Approved

ALABAMA

Alabama remains one of 11 states that have not expanded its Medicaid program under the ACA. In 2013, Alabama received CMS approval to operate Medicaid under a Regional Care Organization (RCO) managed care model, but that effort was abandoned with a change in administration. The state operates several managed care programs for long-term care and pregnant women.

Quick Medicaid Facts

- ***964,161*** *Medicaid Beneficiaries (11/2022)*
- ***FFS Model***
- ***79%*** ***Federally Funded***
- ***Non-Expansion State***
- ***Annual*** *Budget Cycle*
- ***N/A – No MCOs*** *$$$ Rx Management*

Prescription Drug Management

In Alabama, gross spending for drug expenditures before manufacturer rebates in FY2021 totaled $863.6 million, up from $771.5 million in FY2020, a 12 percent increase.

Alabama uses a P&T Committee to advise on Medicaid prescription drug coverage. Specifically, the P&T Committee is responsible for advising on new PDL drugs, while the Medicaid agency assumes leadership on establishing step therapy and prior authorization criteria, and for orphan/expedited review drugs. Reviews for new PDL drugs occur on a quarterly basis while reviews for step therapy and prior authorization review are conducted on an "as needed" basis.

Under current supplementary rebate programs, the Medicaid agency is the primary negotiator.

- PDL for FFS Rx
- N/A No MCOs – PDL for MCO Rx
- Rx Limits – 5 Rx per month (adults) w/ drug class exclusions
- N/A – No MCO
- Generics Promoting Policies in Place

Rx VBP Overview	
SPA #	AL-19-0009 ; AL-20-0021 (amendment)
Date of Approval	CMS Approval on Dec. 20, 2019; Dec. 17, 2020
Proposed Model	Supplemental Rebate Agreement

DISEASE POPULATION IN THE STATE (estimates)

- **Cystic Fibrosis** — ***455***
- **Diabetes** — ***550,149***
- **Hemophilia** — ***100-299***
- **Acute Hepatitis C** — ***49***
- **Sickle Cell Disease** — ***2851***
- **Spinal Muscular Atrophy** — ***183****

**Estimated number of individuals living with SMA*

SPA VBP Approved

ARIZONA

Arizona operates its Medicaid program through the Arizona Health Care Cost Containment System (AHCCCS), a mandatory managed care program that contracts with several MCOs statewide to provide coverage of acute, primary, and specialty care services. Behavioral health services are "carved out" and operated through sub-contracts with the Regional Behavioral Health Authorities (RBHAs), a collection of community-based organizations.

Quick Medicaid Facts

- ***2.1 Million*** *Medicaid Beneficiaries (11/2022)*
- ***FFS/MCO***
- ***76% Federally Funded***
- ***Expansion State*** *September 2015*
- ***Annual*** *Budget Cycle*
- ***Hybrid*** *$$$ Rx Management*

Prescription Drug Management

In Arizona, gross spending for drug expenditures before manufacturer rebates in FY2021 totaled $1.64 billion, up from $1.5 billion in FY2020, a 9 percent increase. FFS and MCO spending accounted for $33 million and $1.61 billion, respectively.

Arizona uses a P&T Committee to advise on Medicaid prescription drug coverage. The P&T Committee is responsible for reviewing new PDL drugs, step therapy criteria, and orphan/ expedited review drugs. Reviews for both new PDL drugs and step therapy criteria occur on an annual basis while reviews for prior authorization criteria are conducted on an "as needed" basis.

- PDL for FFS Rx
- UPDL for MCO Rx – some classes
- No FFS Rx Limits
- Carves In Rx Benefit
- Carves Out Certain Drug Classes
- Generics Promoting Policies in Place

Rx VBP Overview	
SPA #	AZ-19-0004
Date of Approval	CMS Approval on April 28, 2020
Proposed Model	Supplemental Rebate Agreement

DISEASE POPULATION IN THE STATE (estimates)

Cystic Fibrosis	Diabetes	Hemophilia	Acute Hepatitis C	Sickle Cell Disease	Spinal Muscular Atrophy
591	590,916	300-499	N/A	635	245*

*Estimated number of individuals living with SMA

SPA VBP Approved

ARKANSAS

Arkansas expanded Medicaid in January 2014. The Division of Medical Services operates the state Medicaid program under the direction of the Department of Human Services (DSH) through multiple programs, including traditional Medicaid, ARHOME, which uses Medicaid funding to buy private health insurance for beneficiaries, and more specific programs for those with disabilities or certain health conditions. Arkansas contracts with a few MCOs to provide comprehensive services for those with complex behavioral health, developmental, or intellectual disabilities.

Quick Medicaid Facts

- ***992,158*** *Medicaid Beneficiaries (11/2022)*
- ***FFS/MCO***
- ***78%*** ***Federally Funded***
- ***Expansion State*** *January 2014*
- ***Biennial*** *Budget Cycle*
- ***Carve In*** *$$$ Rx Management*

Prescription Drug Management

In Arkansas, gross spending for drug expenditures before manufacturer rebates in FY2021 totaled $404 million, up from $373 million in FY2020, an 8 percent increase. FFS and MCO spending accounted for $320 million and $84 million, respectively.

The Arkansas Medicaid Drug Utilization Review (DUR) board is responsible for making clinical recommendations to the Arkansas Medicaid Pharmacy Program regarding the use of restrictions including prior authorization and re-authorization criteria on prescription drugs covered by Medicaid.

Under the supplementary rebate program, a competitively procured purchasing pool is responsible for negotiations.

- PDL for FFS Rx
- N/A No MCOs -- PDL for MCO Rx
- Rx Limits – 6 Rx per month (adults) w/ drug class exclusions
- Carves in Rx Benefit
- Generics Promoting Policies in Place

Rx VBP Overview	
SPA #	AR-22-0006
Date of Approval	CMS Approval on June 28, 2022
Proposed Model	Supplemental Rebate Agreement

DISEASE POPULATION IN THE STATE (estimates)

Cystic Fibrosis	Diabetes	Hemophilia	Acute Hepatitis C	Sickle Cell Disease	Spinal Muscular Atrophy
301	321,645	100-299	65	1,266	112*

*Estimated number of individuals living with SMA

SPA VBP Approved

COLORADO

Colorado's Medicaid is operated through its Health First Colorado. Colorado operates a primary care case management (PCCM) program that contracts with Regional Care Collaborative Organizations (RCCOs) to provide coverage for acute, physical, and specialty care as well as pharmacy and select behavioral health services. Health First Colorado contracts with two MCOs, one of which is affiliated with UnitedHealth Group. On March 22, 2022, Colorado entered into a VBP contract agreement with Novartis for the drug Zolgensma.

Quick Medicaid Facts

- ***1.6 Million*** *Medicaid Beneficiaries (11/2022)*
- ***FFS/MCO***
- ***56%*** ***Federally Funded***
- ***Expansion State*** *May 2013*
- ***Annual*** *Budget Cycle*
- ***Hybrid*** *$$$ Rx Management*

Prescription Drug Management

In Colorado, gross spending for drug expenditures before manufacturer rebates in FY2021 totaled $1.2 billion, up from $1 billion in FY2020, a 20 percent increase. FFS and MCO spending accounted for $1.1 billion and $44 million, respectively.

Colorado uses a P&T Committee and a DUR Board to advise on Medicaid prescription drug coverage. The P&T Committee is responsible for reviewing new preferred drug list (PDL) drugs, while the DUR Board manages review of step therapy and prior authorization criteria, and orphan/expedited review drugs. Reviews for PDL drug classes are conducted annually, while step therapy and prior authorization criteria reviews vary.

The state established a Prescription Drug Affordability Board tasked with reviewing and setting price limits on prescription medications in June of 2022.

Under current supplementary rebate programs, pharmacy benefit managers (PBMs) are responsible for negotiating supplemental rebates.

- PDL for FFS Rx
- Not Reported -- UPDL for MCO Rx
- No FFS Rx Limits
- Carves In Rx Benefit
- Carves Out Certain Drug Classes
- Generics Promoting Policies in Place

Rx VBP Overview	
SPA #	CO-18-0044
Date of Approval	CMS Approval on Dec. 20, 2019
Proposed Model	Supplemental Rebate Agreement

DISEASE POPULATION IN THE STATE (estimates)

Cystic Fibrosis	Diabetes	Hemophilia	Acute Hepatitis C	Sickle Cell Disease	Spinal Muscular Atrophy
713	311,554	300-499	10	371	196*

*Estimated number of individuals living with SMA

SPA VBP Approved

LOUISIANA

Louisiana's Medicaid is managed through Healthy Louisiana. Healthy Louisiana was created after transforming the former CommunityCARE program into a risk-based comprehensive managed care program. As of January 2023, Healthy Louisiana contracts with six MCOs. On January 1, 2023, the state moved to a single PBM for all MCOs. A single PDL developed by the state remains in place for both FFS and MCO beneficiaries.

Quick Medicaid Facts

- **1.7 Million** *Medicaid Beneficiaries (11/2022)*
- **FFS/MCO**
- **73%** **Federally Funded**
- **Expansion State** *January 2016*
- **Annual** *Budget Cycle*
- **Carve In** *$$$ Rx Management*

Prescription Drug Management

In Louisiana gross spending for drug expenditures before manufacturer rebates in FY2021 totaled $2 billion, up from $1.7 billion in FY2020, an 18 percent increase. FFS and MCO drug spending accounted for $47 million and $1.9 billion, respectively.

Louisiana uses a P&T Committee to advise on Medicaid prescription drug coverage. The P&T Committee is responsible for reviewing new PDL drugs and orphan/expedited review drugs. Reviews for PDL drug classes are conducted on an annual basis, while the timeline for prior authorization criteria reviews are performed on an "as needed" basis.

Under the supplementary rebate program, a competitively procured purchasing pool is responsible for negotiations.

- FFS Rx PDL
- MCO Rx UPDL
- FFS Rx Limits – 4 prescriptions per month
- Carves In Rx Benefit
- Generics Promoting Policies in Place

Rx VBP Overview	
SPA #	LA-19-0018 ; LA-19-0029 (amendment)
Date of Approval	CMS Approval on May 26, 2019; March 11, 2020
Proposed Model	Subscription-Based Model for Hepatitis C Antiviral Agents

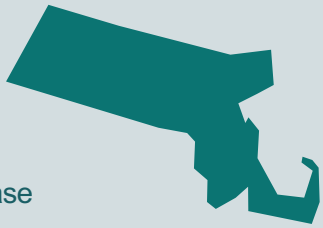
DISEASE POPULATION IN THE STATE (estimates)

Cystic Fibrosis	Diabetes	Hemophilia	Acute Hepatitis C	Sickle Cell Disease	Spinal Muscular Atrophy
366	505,468	100-299	281	3,936	182*

*Estimated number of individuals living with SMA

SPA VBP Approved

MASSACHUSETTS



Massachusetts' Medicaid is managed through MassHealth, which has extended managed care services to Medicaid beneficiaries through the combination of a limited MCO and Primary Care Case Management (PCCM). Under the PCCM model, behavioral health services are carved out of the benefits package but are provided through a mental health pre-paid plan. Through the Senior Care Options (SCO), beneficiaries aged 65 and over are provided with continued coverage for acute, long-term care, and social support services. In 2018, the state announced a major redesign to MassHealth, and automatically shifted most of the benefits under one of 17 contracted accountable care organizations (ACOs). Patients had the flexibility to opt-out of the ACO and enroll in an alternative managed care option.

Quick Medicaid Facts

- ***1.8 Million*** *Medicaid Beneficiaries (11/2022)*
- ***FFS/MCO***
- ***56% Federally Funded***
- ***Expansion State*** *July 2013*
- ***Annual*** *Budget Cycle*
- ***Carve In*** *$$$ Rx Management*

Prescription Drug Management

In Massachusetts gross spending for drug expenditures before manufacturer rebates in FY2021 totaled $1.9 billion, up from $1.6 billion in FY2020, a 19 percent increase. FFS and MCOs drug spending accounted for $970 million and $971 million, respectively.

The Massachusetts Medicaid agency performs the review for new PDL drugs and orphan/ expedited review drugs as well as step therapy and prior authorization criteria. Reviews for all categories are performed on an "as needed" basis.

Under the supplementary rebate program, a competitively procured purchasing pool is responsible for negotiations.

- FFS Rx PDL
- MCO Rx UPDL – for some classes
- No FFS Rx Limits
- Carves In Rx Benefit
- Generics Promoting Policies in Place

Rx VBP Overview	
SPA #	MA-19-0001
Date of Approval	CMS Approval on July 31, 2019
Proposed Model	Supplemental Rebate Agreement

DISEASE POPULATION IN THE STATE (estimates)

Cystic Fibrosis	Diabetes	Hemophilia	Acute Hepatitis C	Sickle Cell Disease	Spinal Muscular Atrophy
840	476,687	300-499	150	1,957	211*

*Estimated number of individuals living with SMA

SPA VBP Approved

MICHIGAN

In Michigan, Medicaid is coordinated through the Michigan Department of Health and Human Services (MDHHS). The state Medicaid agency administers health benefits through two options: 1) traditional Medicaid, and 2) Healthy Michigan Plan. Michigan offers several types of MCO programs, including the Medicaid Health Plans, which offers a managed care pharmacy benefit that contracts with nine MCOs as of March 2023.

Quick Medicaid Facts

2.9 Million
Medicaid Beneficiaries (11/2022)

FFS/MCO

71% **Federally Funded**

Expansion State
April 2014

Annual
Budget Cycle

Hybrid
$$$ Rx Management

Prescription Drug Management

In Michigan, gross spending for drug expenditures before manufacturer rebates in FY2021 totaled $2.8 billion, up from $2.2 billion in FY2020, a 27 percent increase. FFS and MCOs drug spending accounted for $1.3 billion and $1.5 billion, respectively.

To determine Medicaid prescription drug coverage, reviews for new PDL drugs, orphan/ expedited review drugs, and the criteria for step therapy and prior authorization are performed by another state entity. Reviews for all categories are performed on an annual basis.

Under the supplementary rebate program, a pharmacy benefit manager (PBM) is responsible for negotiations.

- PDL for FFS Rx
- No UPDL for MCO Rx
- No FFS Rx Limits
- Carves In Rx Benefit
- Carves Out Certain Drug Classes
- Generics Promoting Policies in Place

Rx VBP Overview	
SPA #	MI-18-0009 ; MI-20-0007 (updates)
Date of Approval	CMS Approval on Nov. 14, 2018; Sep. 28, 2020
Proposed Model	Supplemental Rebate Agreement

DISEASE POPULATION IN THE STATE (estimates)

Cystic Fibrosis	Diabetes	Hemophilia	Acute Hepatitis C	Sickle Cell Disease	Spinal Muscular Atrophy
1,185	912,794	>1,000	119	3,322	330*

*Estimated number of individuals living with SMA

SPA VBP Approved

MISSOURI

In Missouri, Medicaid is coordinated through the Department of Social Services. The state Medicaid agency administers health benefits through two options: 1) the traditional Medicaid program called MO HealthNet, and 2) MO HealthNet Managed Care. As of July 1, 2022. Missouri reported that pharmacy benefits would be carved out of MCO contracts. Currently, the state's Medicaid contracts with three managed care programs.

Quick Medicaid Facts

1.4 Million
Medicaid Beneficiaries (11/2022)

FFS/MCO

66% **Federally Funded**

Expansion State
August 202

Annual
Budget Cycle

Hybrid
$$$ Rx Management

Prescription Drug Management

In Missouri, gross spending for drug expenditures before manufacturer rebates in FY2021 totaled $1.3 billion, up from $1.2 billion in FY2020, an 8 percent increase. FFS drug spending accounted all the spending.

Missouri has a Drug Prior Authorization Committee and a Drug Utilization Review Board that make recommendations and ratify for prior authorization, clinical edit or PDL status.

Under the supplementary rebate program, the state is responsible for negotiations.

- PDL for FFS Rx
- No UPDL for MCO Rx
- No FFS Rx Limits
- Carves Out Rx Benefits in MCOs
- Generics Promoting Policies in Place

Rx VBP Overview	
SPA #	MO-22-0023
Date of Approval	CMS Approval on Jan. 12, 2023
Proposed Model	Supplemental Rebate Agreement

DISEASE POPULATION IN THE STATE (estimates)

Cystic Fibrosis	Diabetes	Hemophilia	Acute Hepatitis C	Sickle Cell Disease	Spinal Muscular Atrophy
784	515,337	300-499	25	1,903	220*

*Estimated number of individuals living with SMA

SPA VBP Approved

NEW YORK

In New York, Medicaid is coordinated through the Department of Health. On April 1, 2023, the pharmacy benefit transitioned from managed care to a FFS program, called NYRx, for all Mainstream Managed Care beneficiaries. This was previously scheduled for implementation on April 1, 2021, but was delayed for two years by the state legislature. This change does not apply to those enrolled in Managed Long-Term Care plans. The scope of benefits will not be changed by this transition.

Quick Medicaid Facts

- ***6.8 Million*** *Medicaid Beneficiaries (11/2022)*
- ***FFS/MCO***
- ***56% Federally Funded***
- ***Expansion State*** *January 2014*
- ***Annual*** *Budget Cycle*
- ***Carve In*** *$$$ Rx Management*

Prescription Drug Management

In New York, gross spending for drug expenditures before manufacturer rebates in FY2021 totaled $6.6 billion, up from $6 billion in FY2020, a 10 percent increase. FFS and MCOs drug spending accounted for $685 million and $5.9 billion, respectively.

Under the NYRx program, a DUR board will review drug classes and make recommendations to the Commissioner of Health on an annual basis regarding the selection of preferred and non-preferred drugs within certain drug classes.

Under current supplementary rebate programs, the Medicaid agency is the primary negotiator.

- PDL for FFS Rx
- UPDL for MCO Rx
- No FFS Rx Limits
- Carves In Rx Benefit
- Generics Promoting Policies in Place

Rx VBP Overview	
SPA #	NY-22-0036
Date of Approval	CMS Approval on April 1, 2022
Proposed Model	Supplemental Rebate Agreement

DISEASE POPULATION IN THE STATE (estimates)

Cystic Fibrosis	Diabetes	Hemophilia	Acute Hepatitis C	Sickle Cell Disease	Spinal Muscular Atrophy
1,684	1.71 Million	>1,000	340	8,661	666*

*Estimated number of individuals living with SMA

SPA VBP Approved

NORTH CAROLINA

North Carolina remains one of 11 states that have not expanded its Medicaid program under the ACA. On March 2, 2023, legislators announced an agreement to expand Medicaid in the state, however, the deal will likely not be voted on for at least a month. On July 1, 2021, North Carolina transitioned to NC Managed Medicaid Care, a system run and managed by five different private insurance companies. Under this program there are two plan options, the Standard Plan and the Tailored Plan, which only select beneficiaries are eligible for, and both plans offer pharmacy benefits.

Quick Medicaid Facts

- **2.0 Million** *Medicaid Beneficiaries (11/2022)*
- ***FFS/MCO***
- **74%** ***Federally Funded***
- ***Non-Expansion State***
- ***Biennial*** *Budget Cycle*
- ***Carve In*** *$$$ Rx Management*

Prescription Drug Management

In North Carolina, gross spending for drug expenditures before manufacturer rebates in FY2021 totaled $2.2 billion, up from $2 billion in FY2020, a 10 percent increase. FFS and MCOs drug spending accounted for $1.8 billion and $330 million, respectively.

North Carolina uses a P&T Committee to advise on Medicaid prescription drug coverage of new PDL drugs and orphan/expedited review drugs, while the Medicaid agency assumes responsibility for establishing step therapy and prior authorization criteria. Reviews for new PDL drugs occur on an annual basis while reviews for step therapy and prior authorization requirements are completed monthly by the P&T Committee.

Under the supplementary rebate program, a purchasing pool is used for negotiation.

- PDL for FFS Rx
- MCO Rx UPDL
- No FFS Rx Limits
- Carves In Rx Benefit
- Generics Promoting Policies in Place

Rx VBP Overview	
SPA #	NC-21-0012
Date of Approval	CMS Approval on Oct 30, 2021
Proposed Model	Supplemental Rebate Agreement

DISEASE POPULATION IN THE STATE (estimates)

Cystic Fibrosis	Diabetes	Hemophilia	Acute Hepatitis C	Sickle Cell Disease	Spinal Muscular Atrophy
1,098	1.01 Million	700-999	75	3,973	371*

*Estimated number of individuals living with SMA

SPA VBP Approved

OHIO

In Ohio, Medicaid is coordinated through the Department of Medicaid. The state contracts with six MCOs, but in October 2022, Ohio carved out components of pharmacy benefits from MCO contracts and began a contract with a single PBM. Additionally, the state is contracting with a Pharmacy Pricing and Audit Consultant (PPAC) for the purposes of support regarding reimbursement, benefit design, oversight, and auditing.

Quick Medicaid Facts

3.1 Million
Medicaid Beneficiaries (11/2022)

FFS/MCO

70% **Federally Funded**

Expansion State
January 2014

Biennial
Budget Cycle

Carve Out
$$$ Rx Management

Prescription Drug Management

In Ohio, gross spending for drug expenditures before manufacturer rebates in FY2021 totaled $3.9 billion, up from $3.4 billion in FY2020, a 15 percent increase. FFS and MCOs drug spending accounted for $291 million and $3.6 billion, respectively.

Ohio uses a P&T Committee to advise on Medicaid prescription drug coverage which is reviewed on an annual basis. The state also utilizes a DUR committee, which reviews consumer claims profiles to determine review criteria, and a board to approve such criteria.

Under current supplementary rebate programs, the state is the primary negotiator.

- PDL for FFS Rx
- MCO Rx UPDL
- No FFS Rx Limits
- Carves In Rx Benefit
- Carves Out Certain Drug Classes
- Generics Promoting Policies in Place

Rx VBP Overview	
SPA #	OH-21-0033
Date of Approval	CMS Approval on Feb. 14, 2022
Proposed Model	Supplemental Rebate Agreement

DISEASE POPULATION IN THE STATE (estimates)

Cystic Fibrosis	Diabetes	Hemophilia	Acute Hepatitis C	Sickle Cell Disease	Spinal Muscular Atrophy
1,616	1.1 Million	>1,000	186	3,725	411*

*Estimated number of individuals living with SMA

SPA VBP Approved

OKLAHOMA

Oklahoma's Medicaid is managed through SoonerCare, which provides coverage for acute, primary, specialty, and behavioral health services. In 2020, the Oklahoma Health Care Authority (OHCA) announced plans to operate SoonerCare under a capitated managed care model, to be implemented in October 2021, but medical plans are yet to be announced. Under this model, which is called SoonerSelect, OHCA expects to provide coverage for pregnant women, children, and newly eligible low-income adults. Oklahoma directly contracts with primary care providers and care coordination services via monthly risk-adjustment case management fees.

Quick Medicaid Facts

- ***1.1 Million*** *Medicaid Beneficiaries (11/2022)*
- ***FFS***
- ***74%*** ***Federally Funded***
- ***Expansion State*** *July 2021*
- ***Annual*** *Budget Cycle*
- ***N/A – No MCOs*** *$$$ Rx Management*

Prescription Drug Management

In Oklahoma, gross spending for drug expenditures before manufacturer rebates in FY2021 totaled $575 million, up from $532 million in FY2020, an 8 percent increase. FFS drug spending accounts for all drug costs.

Oklahoma uses a DUR Board for reviews of new PDL drugs, orphan/expedited review drugs, and step therapy and prior authorization requirements. Reviews for PDL drug classes are conducted on an "as needed" basis, while step therapy and prior authorization criteria reviews are completed annually.

Under the supplementary rebate program, a competitively procured purchasing pool is responsible for negotiations.

- PDL for FFS Rx
- No UPDL for MCO Rx
- FFS Rx Limits – 6 prescriptions per month w/ certain exclusions
- N/A – No MCOs
- Generics Promoting Policies in Place

Rx VBP Overview	
SPA #	OK-18-0008
Date of Approval	CMS Approval on June 27, 2018
Proposed Model	Supplemental Rebate Agreement

DISEASE POPULATION IN THE STATE (estimates)

Cystic Fibrosis	Diabetes	Hemophilia	Acute Hepatitis C	Sickle Cell Disease	Spinal Muscular Atrophy
364	373,824	100-299	20	753	151*

*Estimated number of individuals living with SMA

SPA VBP Approved

PENNSYLVANIA

Pennsylvania's Medicaid program, also known as Medical Assistance (MA), is coordinated through the Department of Human Services, which administers the pharmacy benefit for beneficiaries covered under the FFS program. Additionally, MA contracts and is operated through nine statewide managed care organizations. The statewide PDL applies to beneficiaries of two of the MCOs, HealthChoices and CommunityHealthChoices.

Quick Medicaid Facts

- ***3.9 Million*** *Medicaid Beneficiaries (11/2022)*
- ***FFS/MCO***
- **58%** ***Federally Funded***
- ***Expansion State*** *January 2015*
- ***Annual*** *Budget Cycle*
- ***Carve In*** *$$$ Rx Management*

Prescription Drug Management

In Pennsylvania, gross spending for drug expenditures before manufacturer rebates in FY2021 totaled $3.8 billion, up from $3.2 billion in FY2020, a 19 percent increase. FFS and MCOs drug spending accounted for $25 million and $3.7 billion, respectively.

The P&T Committee in the state acts in an advisory capacity to provide clinical recommendations on the statewide PDL which must then be reviewed and approved by the Secretary of the Department of Human Services. Additionally, the state utilizes a DUR Board.

Under current supplementary rebate programs, the state is the primary negotiator.

- PDL for FFS Rx
- UPDL for MCO Rx
- No FFS Rx Limits
- Carves In Rx Benefit
- Generics Promoting Policies in Place

Rx VBP Overview	
SPA #	PA-22-0005
Date of Approval	CMS Approval on Aug. 19, 2022
Proposed Model	Supplemental Rebate Agreement

DISEASE POPULATION IN THE STATE (estimates)

Cystic Fibrosis	Diabetes	Hemophilia	Acute Hepatitis C	Sickle Cell Disease	Spinal Muscular Atrophy
1,578	*1.1 Million*	*>1,000*	*146*	*3,743*	*415**

*Estimated number of individuals living with SMA

SPA VBP Approved

TENNESSEE

Tennessee Medicaid, also known as TennCare, is administered by the Division of TennCare. Under the SPA for the supplemental rebate agreement, TennCare moved to a single, statewide PDL for the entire pharmacy program. Additionally, TennCare employs a single PBM to process all TennCare pharmacy claims and respond to all prior approval requests through OptumRx. Pharmacy benefits are generally carved out of MCO contracts and are instead provided by Pharmacy Benefit Administrators contracted with the state. Currently, the state's Medicaid contracts with three managed care programs.

Quick Medicaid Facts

- ***1.6 Million*** *Medicaid Beneficiaries (11/2022)*
- ***FFS/MCO***
- ***65% Federally Funded***
- ***Non-Expansion State***
- ***Annual*** *Budget Cycle*
- ***Hybrid*** *$$$ Rx Management*

Prescription Drug Management

In Tennessee, spending for pharmacy benefits in FY2021 totaled $1.3 billion, up from $1.2 billion in FY2020, an 8 percent increase. FFS and MCOs drug spending accounted for $1.2 billion and $130 million, respectively. Tennessee generally carves out prescription drugs from its MCO program, but MCO spending may reflect physician-administered drugs.

A prospective drug utilization review is run through OptumRx that encompasses the detection, evaluation, and counseling components of predispensing drug therapy screening.

Under the supplementary rebate program, the state is responsible for negotiations.

- PDL for FFS Rx
- N/A No MCOs Rx-- PDL for MCO Rx
- FFS Rx Limits – 5 prescriptions per month (adults)
- Carves Out MCO Rx Benefits
- Generics Promoting Policies in Place

Rx VBP Overview	
SPA #	TN-21-0004
Date of Approval	CMS Approval on Aug. 18, 2021
Proposed Model	Supplemental Rebate Agreement

DISEASE POPULATION IN THE STATE (estimates)

Cystic Fibrosis	Diabetes	Hemophilia	Acute Hepatitis C	Sickle Cell Disease	Spinal Muscular Atrophy
811	760,719	300-499	170	2,077	250*

*Estimated number of individuals living with SMA

SPA VBP Approved

TEXAS

Texas Medicaid is operated by the Texas Medicaid and Healthcare Partnership (TMHP) and managed through the STAR managed care program, which delivers coverage through managed care plans under contract with the state. In those programs, patients receive services through chosen health plans. Members of this program get Medicaid benefits in addition to add-on services as needed for an additional, but discounted rate. In addition to STAR, Texas operates two other managed care programs: STAR+PLUS and STAR Health. Texas contracts with multiple plans comprised of local non-profit plans and national for-profit plans.

Quick Medicaid Facts

- ***5.4 Million*** *Medicaid Beneficiaries (11/2022)*
- ***FFS/MCO***
- **66%** ***Federally Funded***
- ***Non-Expansion State***
- ***Biennial*** *Budget Cycle*
- ***Hybrid*** *$$$ Rx Management*

Prescription Drug Management

In Texas, spending for pharmacy benefits in FY2021 totaled $3.3 billion, up from $3.2 billion in FY2020, a 3 percent increase. FFS and MCOs drug spending accounted for $55 million and $3.29 billion, respectively.

Texas uses a DUR Board for reviews of new PDL drugs and step therapy and prior authorization criteria, while the Medicaid agency manages orphan/expedited review drugs. Reviews are conducted on a quarterly basis. Recommendations are forwarded to the Texas Medicaid agency.

Under the supplementary rebate program, a competitively procured external vendor is responsible for negotiations.

- PDL for FFS Rx
- UPDL for MCO Rx
- FFS Rx Limits – 3 prescriptions per month (adults)
- Carves In Rx Benefit
- Carves Out Certain Drug Classes
- Generics Promoting Policies in Place

Rx VBP Overview	
SPA #	TX-20-0010
Date of Approval	CMS Approval on Sept. 28, 2020
Proposed Model	Supplemental Rebate Agreement

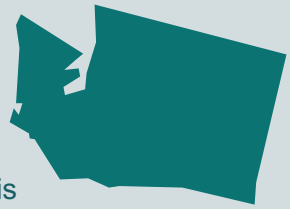
DISEASE POPULATION IN THE STATE (estimates)

Cystic Fibrosis	Diabetes	Hemophilia	Acute Hepatitis C	Sickle Cell Disease	Spinal Muscular Atrophy
2,223	2.7 Million	> 1,000	17	7,132	1,164*

*Estimated number of individuals living with SMA

SPA VBP Approved

WASHINGTON



Washington's Medicaid is managed through the Apple Health program. Apple Health contracts and is operated through five statewide managed care organizations. In addition to its health managed care program, the state also operates two other managed care delivery programs for behavioral health and long-term care – the Regional Support Networks (RSN) model, a joint 11 county-based collaborative, and the All-Inclusive Care for the Elderly (PACE) program.

Quick Medicaid Facts

- ***2.1 Million*** *Medicaid Beneficiaries (11/2022)*
- ***FFS/MCO***
- ***56%*** ***Federally Funded***
- ***Expansion State*** *June 2013*
- ***Biennial*** *Budget Cycle*
- ***Hybrid*** *$$$ Rx Management*

Prescription Drug Management

In Washington, spending for pharmacy benefits in FY2021 totaled $1.4 billion, up from $1.2 billion in FY2020, a 17 percent increase. FFS and MCOs drug spending accounted for $86 million and $1.3 billion, respectively.

Reviews of the criteria for step therapy and prior authorization, and orphan/expedited review drugs are performed by the Medicaid agency, while another state entity carries out reviews for the PDL. All reviews are completed on an annual basis.

Under the supplementary rebate programs, multiple competitively procured entities are responsible for negotiations.

- PDL for FFS Rx
- UPDL for MCO Rx – for some classes
- No FFS Rx Limits
- Carves In Rx Benefit
- Carves Out Certain Drug Classes

Rx VBP Overview	
SPA #	WA-19-0008
Date of Approval	CMS Approval on June 12, 2019
Proposed Model	Subscription-Based Model for Hepatitis C Antivirals

DISEASE POPULATION IN THE STATE (estimates)

Cystic Fibrosis	Diabetes	Hemophilia	Acute Hepatitis C	Sickle Cell Disease	Spinal Muscular Atrophy
723	582,006	300-499	105	370	264*

*Estimated number of individuals living with SMA

About The Campaign for Transformative Therapies

The Campaign for Transformative Therapies (CTT) is an issue-driven campaign of the Council for Affordable Health Coverage (CAHC) that brings together diverse interests - including organizations representing insurers, drug manufacturers, and patient groups. We support policies that encourage outcomes-based arrangements for gene therapies to ensure patient access.

A CAMPAIGN FOR Transformative Therapies